

108TH CONGRESS  
1ST SESSION

# H. R. 894

To amend the Public Health Service Act to require the Director of the National Institutes of Health to expand and intensify research regarding Diamond-Blackfan Anemia.

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## IN THE HOUSE OF REPRESENTATIVES

FEBRUARY 25, 2003

Mrs. MCCARTHY of New York introduced the following bill; which was referred to the Committee on Energy and Commerce

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## A BILL

To amend the Public Health Service Act to require the Director of the National Institutes of Health to expand and intensify research regarding Diamond-Blackfan Anemia.

1       *Be it enacted by the Senate and House of Representa-*  
2       *tives of the United States of America in Congress assembled,*

3       **SECTION 1. SHORT TITLE.**

4       This Act may be cited as the “Diamond-Blackfan  
5       Anemia Act”.

6       **SEC. 2. FINDINGS.**

7       The Congress finds as follows:

1           (1) Diamond-Blackfan Anemia (in this section  
2           referred to as “DBA”) is a rare genetic bone mar-  
3           row failure disorder, which develops in infancy and  
4           results in severe anemia due to failure to make red  
5           blood cells.

6           (2) Treatments for DBA, including the use of  
7           steroids (such as prednisone) and blood transfusions,  
8           have potential long-term side effects, including  
9           osteoporosis, impaired growth because of the  
10          steroids, and iron overload because of the trans-  
11          fusions. DBA patients have an increased risk of leu-  
12          kemia, solid tumors, and complete bone marrow fail-  
13          ure.

14          (3) The only cure for DBA is a bone marrow  
15          transplant, a procedure that carries serious risks  
16          and, since most patients lack an acceptable donor, is  
17          an option for only about 25 percent of patients.

18          (4) Although there are a few studies focusing  
19          on other bone marrow failure syndromes and one in-  
20          tramural study investigating the efficacy of stem cell  
21          treatment for DBA patients, there are almost no  
22          studies conducted at or with extramural funding  
23          from the National Institutes of Health that are spe-  
24          cifically aimed at the identification, treatment, and  
25          cure of DBA.

1 **SEC. 3. DIAMOND-BLACKFAN ANEMIA.**

2 The Public Health Service Act is amended by insert-  
3 ing after section 424B (42 U.S.C. 285b-7b) the following:

4 “DIAMOND-BLACKFAN ANEMIA

5 “SEC. 424C. (a) IN GENERAL.—The Director of the  
6 Institute, in coordination with the National Heart, Lung,  
7 and Blood Institute and the Office of Rare Diseases, shall  
8 expand and intensify research and related activities of the  
9 Institute with regard to Diamond-Blackfan Anemia.

10 “(b) PROGRAMS.—In carrying out subsection (a):

11 “(1) SCIENTIFIC WORKSHOP.—The Director of  
12 the Institute shall convene a scientific workshop for  
13 the following:

14 “(A) Evaluation of the state of research on  
15 Diamond-Blackfan Anemia.

16 “(B) Determination of areas of importance  
17 for such research (which may include red cell  
18 differentiation, ribosomal protein function, can-  
19 cer predisposition, and gene discovery).

20 “(C) Development of a comprehensive plan  
21 for conducting and supporting such research.

22 “(2) RESEARCH.—In accordance with the com-  
23 prehensive plan developed under subsection  
24 (b)(1)(C), the Director of the Institute shall conduct  
25 or support research on the following:

1                   “(A) The pathophysiology of Diamond-  
2                   Blackfan Anemia.

3                   “(B) The relationship between Diamond-  
4                   Blackfan Anemia and predisposition to cancer.

5                   “(C) Developing better treatments and ul-  
6                   timately a cure for Diamond-Blackfan Anemia.

7                   “(3) DATABASE.—The Director of the Institute  
8                   shall facilitate the expansion and maintenance of, as  
9                   well as foster the use of, the Diamond-Blackfan  
10                  Anemia National Registry.”.

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